

Good afternoon! My name is Colleen Labbadia and I am honored to be here today to represent Parent Project Muscular Dystrophy and the entire Duchenne muscular dystrophy community. My 4-year old son has Duchenne, a 100% fatal neuromuscular condition that is caused by the lack of a protein called dystrophin. It is a serious condition that causes progressive muscle weakness and life expectancy only into the twenties. There is currently no cure!

In order to give those with Duchenne a fighting chance at a possible treatment, we are in desperate need of three regulatory changes:

#1 – The need for better guidance between FDA and Sponsors. A significant hurdle for Duchenne in drug development is the absence of clear guidance from the FDA when it comes to designing clinical trials. Small patient populations and a lack of accepted or validated biomarkers (that indicate a potential treatment is working) are some of our major challenges.

This June, in an effort to reduce the existing barrier in drug development, PPMD and a broad coalition of stakeholders submitted to the FDA, the first-ever patient advocacy-initiated draft guidance for a rare disease to help accelerate development and review potential therapies for Duchenne.

#2 – The need for better outcome measures and biomarkers. Currently, the only validated marker in Duchenne is the 6-minute walk test. This marker leaves a narrow window of boys eligible to be tested in trials. The very young are excluded due to lack of ability to complete the 6MWT and the non-ambulatory population is excluded because of their inability to take the test, along with the lack of validated alternative measures that could test upper extremities, cardiac, or respiratory functions. Essentially, once boys go off their feet, it leaves families and patients hopeless for any options to participate in trials.

Each loss of function for our boys, including my son, is a "little death"... it is loss of function that he will not get back. The ripple affect from this is devastating. The negative impact on the family and the burden on the caregiver becomes increasingly significant and grows over time.

#3 – The need for increased flexibility by the FDA and the incorporation of patient preferences. Our community needs more flexibility with clinical trial designs and reviews. The standard approach to drug development used in more common diseases has, to date, failed to deliver even modest therapeutic options for patients with Duchenne (and other rare diseases). The current system is not working for our boys. The full benefit of many of these drugs cannot be seen in the standard 48 week period of a trial.

The FDA must utilize the tools that Congress has given them, particularly within the new FDASIA law where the accelerated approval pathway was modernized. Patients

are waiting so the use of this pathway for diseases like Duchenne is crucial and time sensitive.

The FDA must also incorporate the benefit risk preference of the Duchenne community into their decision-making. Parents, like myself, are willing to accept significant risks for a treatment that may merely just slow the progression of Duchenne. Any hope is better than none.

Overall, time matters for the Duchenne community. As we wait for these regulatory changes and advancements, our boys are slowly losing abilities that most of us take for granted. These are abilities that will not return. Working through the current regulatory issues and challenges that plague our community, would be advantageous for all boys with Duchenne, including my brave little guy. Now is the time for change. Now is the time to help these boys and give them the bright future that they deserve!